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5630 Fishers Lane
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Submitter: Gregory C. Simon
Organization: *FasterCures / The Center for Accelerating Medical Solutions*

Re: Solicitation of Comments on Stimulating Innovation in Medical Technologies
Docket No. 2004S-0233

Dear Madam/Sir:

FasterCures is a nonpartisan, nonprofit organization whose goal is to save lives by saving time in the discovery, development, and deployment of treatments and cures for deadly diseases. Our mission is to evaluate the current system of medical research; identify inefficiencies, misplaced priorities, and conflicting incentives inhibiting the pace of discovery and development; and propose and pursue improvements to the existing system. We seek to enhance and accelerate the efforts of those involved in developing and overseeing the introduction of safe and effective treatments and cures.

As such, we share the Department of Health and Human Services' (HHS) goal of accelerating biomedical discovery and its translation into new treatments and cures. We appreciate this opportunity to provide our view of several approaches that could be taken by HHS and its agencies to coordinate efforts aimed at stimulating medical technology innovation.

Despite the remarkable advances medical research has produced in recent years, cancer, heart disease, diabetes, and other illnesses continue to take a staggering toll in treatment costs, lost productivity, suffering, and death. By 2001, healthcare spending accounted for more than 14 percent of the gross domestic product. With 76 million baby-boomers turning 50 at the rate of one every seven seconds, it is imperative that we move research advances forward more quickly. As our population ages, the number of those afflicted by disease and the costs to treat these individuals will rise exponentially in the decades ahead.

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At the same time that our nation's demographics will create new challenges to our healthcare systems, the biomedical revolution – combined with rapid technological innovation – presents the promise of longer life spans and greater relief from suffering. However, there are numerous indications that the massive United States investment in biomedical research and discovery is not being translated as rapidly as possible into new medicines and treatments. With the mandate of protecting and promoting the health of all Americans, HHS must lead the transformation of our current research and healthcare system from the outdated model of the last century to an integrated, information-based, high-quality, health-sustaining model that will extend life expectancy and improve the quality of life in the 21st century.

THE CALL FOR A NATIONAL COMMITMENT TO FINDING CURES AND TREATMENTS FOR SERIOUS AND DEADLY DISEASES

It is no secret that a major obstacle to faster cures is the lack of cooperation and coordination among the institutions and organizations responsible for supporting the medical research system. However, the challenge of bringing all the available resources to bear on finding faster cures will not be met simply by better coordination, nor is it only about the amount of money being spent on the effort. This commitment should focus on maximizing the return of investment on every research dollar, establishing clearly articulated and coordinated goals, and demanding accountability for accomplishing these goals.

In our view, the President, with the support of the Secretary of Health and Human Services (the Secretary) must make a national commitment to finding treatments and cures for serious and deadly diseases, and provide the leadership and resources necessary to translate today's scientific and technological advances into new therapies. Time and time again our nation has responded to great national challenges: overcoming daunting odds and seemingly insurmountable obstacles. But it takes a commitment of national will and the leadership to motivate and inspire participation from all segments of society. It is now time to declare war on the diseases that shorten and impair the lives of millions of people each year.

FasterCures is strongly supportive of and encouraged by various HHS initiatives already underway, including: the Food and Drug Administration's (FDA) Critical Path Initiative; the National Institutes of Health (NIH) Roadmap Initiative; the Futures Initiative of the Centers for Disease Control and Prevention (CDC); the Translating Research into Practice (TRIP) program of the Agency for Healthcare Research and Quality (AHRQ); and HHS' National Health Information Infrastructure (NHII) Initiative. However, the potential for these programs will only be fully realized to the extent that these individual agency initiatives are organized and directed toward an overarching goal, with HHS providing leadership, coordination, resources, and accountability. It is not enough, for example, for the National Cancer Institute (NCI) to commit to eliminating suffering and death due to cancer – a commitment which *FasterCures* fully supports -- if the infrastructure necessary to translate those discoveries into treatments is lacking and the process to do so takes an additional 17 years.

In *FasterCures'* view, creating a mechanism to facilitate cooperation, set priorities, establish performance goals and measures, and maintain accountability across HHS agencies is critical to the success of this mission. As a first step, *FasterCures* recommends that HHS immediately convene an **HHS Interagency Task Force**, reporting directly to the Secretary, to coordinate, develop and implement interagency innovation initiatives. The Secretary should allocate funds from the current HHS budget and, as necessary, pursue additional authorizing and budget authority to support these initiatives. The Secretary also should pursue, as necessary, other transactional authority that would allow HHS and its agencies to employ innovative procurement, acquisition, and public/private partnership strategies similar to those available to the Department of Defense (DOD) and the Central Intelligence Agency (CIA).

A coordinated leadership effort, as envisioned in the proposed **HHS Interagency Task Force,** is essential to successfully stimulating innovation in medical technology in the most efficient and effective way possible. It also is a critical component of our recommendations.

FasterCures congratulates Secretary Thompson and all of HHS for your efforts to stimulate innovation in medical research and technologies. At *FasterCures,* our mission is to saves lives by finding ways to save time in the discovery and development of new medical therapies, while at the same time assuring patient safety, protecting privacy and maintaining the highest level of quality and integrity in the research and development process. We are committed to working with HHS and other partners in the government, industry, advocacy, and academic sectors to realize that potential. We would welcome the opportunity to work with HHS in developing and implementing programs to stimulate innovation and remove the obstacles to progress in the discovery and development of new medical therapies for serious and deadly diseases.

Thank you for the opportunity to provide these comments.

Gregory C. Simon, JD

President, *FasterCures / The Center for Accelerating Medical Solutions*

RESPONSE TO THE DEPARTMENT OF HEATLH AND HUMAN SERVICES REQUEST FOR COMMENTS ON STIMULATING INNOVATION IN MEDICAL TECHNOLOGIES DATED MAY 24, 2004

SUMMARY OF *FASTERCURES'* RECOMMENDATIONS

In summary, *FasterCures'* specific recommendations to HHS include the following:

- Convene an HHS interagency task force reporting directly to the Secretary, to develop and implement interagency innovation initiatives.

- Improve the clinical trials process by:
  - Optimizing clinical trial design and eliminating redundancy (NIH, FDA, CMS, HHS);
  - Standardizing clinical trial data management, analysis, and reporting (NIH, FDA);
  - Developing standardized biomarkers and surrogate endpoints (NIH, FDA);
  - Increasing the public's understanding of the clinical trial system and how to participate (FDA, NIH);
  - Reimbursing medical expenses incurred by participants in NIH-supported clinical trials (NIH, CMS); and
  - Ensuring reimbursement for patient-incurred costs associated with prevention research (NIH, CDC, FDA, CMS).

- Enhance clinical research tools and funding mechanisms by:
  - Expanding access to and sharing of research data (NIH, FDA, CMS, CDC, HHS);
  - Developing innovative funding models to support large-scale, interagency research and facilitate public-private research partnerships (NIH, CDC, HRSA, HHS); and
  - Supporting clinical research training and professional development (HHS, CDC, FDA, NIH).

- Facilitate and ensure patient/subject and privacy protection by:
  - Harmonizing regulatory requirements related to human subject protections (FDA, NIH, HHS);
  - Using a modified version of the NCI's informed consent template to create a simplified informed consent template (FDA, HHS);
  - Streamlining the Institutional Review Board (IRB) process for large multi-center clinical trials (HHS, NCI, NIH, FDA); and
  - Clarifying the applicability of the Health Insurance Portability and Accountability Act (HIPAA) to medical research contexts, and harmonizing HIPAA privacy and confidentiality provisions with other human subject research regulations (NIH).

- Facilitate the emergence of "personalized medicine" through development of appropriate regulatory approaches.

Summary of Recommendations, cont.

- Explore ways to modify the current federal reimbursement structure to reward innovation.

- Involve all relevant stakeholders in the development of initiatives addressing existing barriers to innovation, with targeted outreach to communities currently underrepresented in medical research.

- Optimize the portability of information between HHS agencies:
  - Require greater data sharing and the creation of interoperable databases among and within HHS agencies (all HHS Agencies);
  - Support programs to increase data sharing within and between the industry, government and academic communities (NIH, FDA); and
  - Fully integrate medical research needs into the NHII (HHS).

- Enlist non-governmental communities in innovation efforts:
  - Partner with academic training institutions to ensure the availability of an adequate supply of clinical researchers by creating new incentives to promote interest in clinical research;
  - Initiate public education programs with voluntary health associations, disease foundations, and public charities about clinical trial participation and programs to increase clinical trial enrollment; and
  - Initiate innovative research collaborations to conduct targeted, disease-specific research in partnership with disease foundations.

FASTERCURES' RECOMMENDATIONS IN RESPONSE TO THE DEPARTMENT OF HEALTH AND HUMAN SERVICES REQUEST FOR COMMENTS ON STIMIULATING MEDICAL INNOVATION DATED MAY 24, 2004

1. *WHAT STRATEGIES AND APPROACHES COULD HHS IMPLEMENT TO ACCELERATE THE DEVELOPMENT AND APPLICATION OF NEW MEDICAL TECHNOLOGIES?*

   and

2. *HOW CAN HHS HELP ITS AGENCIES (E.G., NIH AND ITS GRANTEES, FDA, CDC, AND CMS) TO WORK TOGETHER MORE EFFECTIVELY TO ELIMINATE OBSTACLES TO DEVELOPMENT OF MEDICAL TECHNOLOGIES?*

HHS should convene an interagency task force, reporting directly to the Secretary, to coordinate, develop, and implement specific innovation initiatives within a one-, two- and three-year timeframe. HHS must provide the leadership, resources, and accountability to ensure that all of its constituent agencies prioritize innovation initiatives. Through the proposed task force, HHS can provide the organizational structure through which agencies would work in concert to define, prioritize, and achieve specific innovation goals. HHS should require that specific goals and timetables be developed for all innovation projects. The Secretary should solicit input, support, and cooperation from Congress and other stakeholders in the biomedical research community.

Working groups from the task force should address the following issues: improving the clinical trials process; enhancing clinical research tools and funding mechanisms; facilitating and ensuring patient/subject and privacy protections; facilitating the development of "personalized medicine" through developing appropriate regulatory approaches; modifying the current federal reimbursement structure so as to encourage innovation; and facilitating stakeholder participation in the design and implementation of innovation initiatives. Each topic is discussed below.

Improve the Clinical Trials Process

A coordinated effort by HHS could make a significant difference in the development of new medical technologies by improving the clinical trials process. Numerous studies have identified inefficiencies in this process as one of the most significant barriers to therapeutic innovation. There are several ways to improve the process:

- **Optimize clinical trial design and find ways to eliminate redundancy. (NIH, FDA, CMS, HHS)**

HHS should expand efforts to optimize clinical trial design and develop coordination tools to eliminate redundancy and improve efficiency. The NIH has begun working to address these issues through Roadmap initiatives such as the Regional Translational Research Centers (RTRCs) and the Integration of Clinical Research Networks. The use of clinical trial networks

already has proven beneficial in instances where multiple interventions could be addressed by trials with similar structure (e.g., patient cohorts, similar protocols and procedures, data collection forms and processing). The FDA and the NIH already have worked together to develop innovative clinical trial designs – such as the Digital Mammography study.

HHS should promote partnerships between the NIH, FDA, CDC, non-profits, and academic and industry scientists to develop better disease models. An improved understanding of the course of disease over time could have profound impacts on the medical technology innovation process by improving the predictive power of preclinical testing, thereby reducing the risk inherent in novel therapeutic development. Targeted research on better disease models already has proved successful in developing effective countermeasures for bioterrorism. As such research progresses, the FDA and the NIH should be prepared to rapidly incorporate new models into the therapeutic development process.

Incorporation of information and tele-health technologies and data management tools could improve the communication and efficiency of doctors conducting clinical trials at multiple sites and streamline data collection, documentation, and regulatory compliance. The use of tele-health technologies also could facilitate the enrollment of participants by reducing the number of visits required to often-distant clinical trial sites. CMS and the FDA should consider developing incentives (such as reimbursements and expedited review) to encourage more widespread use of such tools.

Additionally, AHRQ – while evaluating the utility of information technology tools for enhancing treatment and healthcare delivery – could assess the impacts of the use of such tools within the research context (e.g., impacts on clinical trial enrollments, time to trial completion, effects on regulatory compliance, and other potential outcome measures). This evaluation could provide useful cost-benefit information to support expanded use of these tools.

Finally, HHS should support and expand ongoing efforts to harmonize international standards related to drug development and review, while ensuring that standards necessary to protect public health and safety are maintained. Increasingly, medical research and development are occurring on a global scale. Clinical development and testing programs in particular are being launched in developing and newly industrialized countries. In particular, harmonization of clinical trial requirements can help to prevent duplication of clinical trials and promote efficiency.

- **Standardize clinical trial data management, analysis, and reporting. (NIH, FDA)**

The clinical trials methodologies used by investigators are described by some as a "Tower of Babel," in which protocol format, clinical endpoints, data collection forms, informed consent, toxicity criteria, and degree of computerization of data differ among organizations and federal agencies. Developing greater uniformity in clinical trial data collection will enable greater coordination among trial centers, easier regulatory review, and enhanced ability to pool and compare clinical trial data. Standardization also will lower barriers to computerization of data, facilitating more rapid data analysis and discovery of adverse events.

Common toxicity data criteria should be developed in order to overcome the complexity of the current system of toxicity tables. Uniform toxicity criteria across all studies would provide comparability across the system, easing data analysis. Finally, common biostatistical principles should be developed for use in evaluating data such as endpoints and sample size. These standards should be developed through collaboration between the FDA and the NIH's RTRCs and piloted within NIH's GCRCs.

- **Develop standardized biomarkers and surrogate endpoints. (NIH, FDA)**

Increased use of surrogate endpoints has the potential to speed up drug approval for treatments that might otherwise have to wait years for clinical endpoint data to be collected, such as morbidity and mortality. Furthermore, the availability of additional validated surrogate endpoints and biomarkers for disease progression might enable drug companies to design shorter clinical trials and target drugs more appropriately to specific populations.

Identification and validation of new surrogate endpoints and biomarkers should be a priority. The NIH and the FDA should bring disease researchers together to reach consensus regarding which biomarkers and surrogate endpoints are the most promising to pursue. Finally, the FDA has indicated that it intends to develop a model for the regulatory acceptance of certain genomic biomarkers. *FasterCures* recommends this process be expanded to include other types of biomarkers.

- **Facilitate greater voluntary and informed public participation in clinical trials and medical research. (FDA, NIH)**

Clinical trial enrollment is one of the slowest and costliest steps in the drug development process. A 2001 Harris Interactive survey found that 85 percent of cancer patients were either unaware or unsure that participation in a clinical trial could be an option for them. HHS should take immediate steps to ensure that the public, and particularly healthcare providers, are fully informed about what clinical trials are available and open for enrollment. For trials that are related to treatments for serious and life-threatening disease, HHS should take further steps to conduct outreach to eligible patients. This outreach could include providing detailed information about available sources of reimbursement or other assistance, expanded information to help patients and their physicians determine eligibility for particular trials, and providing assistance and training for physicians who might want to participate as researchers.

HHS should establish a partnership among the NIH, FDA, CMS, the National Library of Medicine, medical professional societies, patient advocacy organizations, and industry organizations to conduct education and outreach about the need for and benefits of patient participation in clinical trials and to identify and remove obstacles to clinical trial participation.

- **Reimburse medical expenses incurred by patients in NIH-supported clinical trials. (NIH, CMS)**

Lack of third-party reimbursement for clinical trials is a critical barrier to patient participation. HHS, the NIH, and CMS should expand the current programs that provide for reimbursement of medical expenses related to clinical trial participation to all NIH-sponsored or funded clinical trials related to serious and life-threatening disease – including prevention and detection trials. Two models for potential CMS coverage of clinical trials are the DOD TRICARE program and the Department of Veterans Affairs (VA) program where beneficiaries can participate in NCI-sponsored cancer clinical trials as a part of their health benefits. The agreements between these agencies and the NCI cover the costs of either NCI-sponsored prevention and detection trials (DOD) or NCI-sponsored prevention, diagnostic, and Phase I-III treatment trials (VA).

- **Ensure reimbursement for patient-incurred costs associated with prevention research. (NIH, CDC, FDA, CMS)**

Disease prevention is fast becoming a critical component of medical innovation. As highlighted in the NCI's 2015 initiative, disease prevention tools will be some of the most important therapeutic discoveries of the future. *FasterCures* supports the Secretary's interest in promoting more health screening and encouraging physical activity. We recommend increased partnering among the NIH, CDC, and the FDA to support research on the role of nutrition in disease prevention and create better health and early-stage disease models. At the same time, CMS should develop reimbursement mechanisms that incentivize disease prevention.

Enhance Clinical Research Tools and Funding Mechanisms

- **Expand access to and sharing of research data. (NIH, FDA, CMS, CDC, HHS)**

Limited access to data generated from research and development is a major obstacle preventing the development of faster cures across all medical research. A significant amount of the data developed from the investment of billions of dollars in biomedical research by both the public and private sectors is neither publicly available nor broadly accessible. Typically this information is stored in proprietary, stand-alone databases that are intentionally kept private and often have little or no interoperability with other databases. Broad data access could reduce some of the redundancy inherent in the research process and stimulate innovation by allowing for increased analysis and pooling of data.

Specific recommendations about how to achieve these goals are addressed under our response to Question #5 below.

- **Develop innovative funding models to support large scale, interagency research and facilitate public-private research partnerships. (NIH, CDC, HRSA, HHS)**

We increasingly are moving into an era of large-scale population-based projects that will cut across agency missions and likely require multiple funding sources. Novel funding mechanisms for large-scale inter-agency clinical research initiatives must be developed.

For example, the NIH, CDC, and the Health Resources and Services Administration (HRSA) have been discussing the need for and a scientific approach to a large population-based study that would examine genotype/phenotype correlations across a normal population. Such an evidence base could provide information crucial to translating genomic information into clinical application. It would require the collection of as much as 1 million samples and accompanying medical information from the U.S. population. Such a study would be very costly, potentially requiring funding by multiple agencies, even beyond the HHS, and requiring support from the private sector. Given the potential benefits from this type of study, funding options should be created by HHS and its agencies to make such studies possible, and partnerships with the private sector should be facilitated.

HHS also should pursue authority that would allow it to create innovative research and development initiatives similar to those that have been established to address defense and intelligence research and development needs. Such authority would provide exemptions from federal procurement and acquisition requirements that pose obstacles to funding of targeted, private sector research and development. In addition to adopting such innovative funding models, HHS should implement improved and widespread auditing procedures to gauge and increase the efficiency of government-funded biomedical and clinical research.

- **Support clinical research training and professional development. (HHS, CDC, FDA, NIH)**

Both the FDA's Critical Path Report and the NIH's Roadmap Initiative identify a well-trained, highly skilled clinical research workforce as a central national research and innovation need. The availability of training programs and opportunities for professional advancement are important incentives to increase workforce participation in a particular field.

HHS should develop incentives to support clinical research training and professional development. Attracting prospective clinical researchers to the field and retaining existing experts will require changes to the current system. Changes could include expanding such programs as the NIH's educational-loan-relief program – which provided $30 million to eligible clinical researchers – and developing training modules and funding mechanisms to enable scientists to transition from basic to clinical research.

HHS should explore new public-private partnerships to promote training and experience in clinical research. Both the FDA and the CDC hold cross-cutting and unique clinical research knowledge invaluable to both training and professional development.

Facilitating and Ensuring Patient/Subject and Privacy Protections

HHS' effort to drive medical technology innovation must remain firmly grounded in its obligation to protect patient health and privacy and the rights and welfare of research subjects. Such protections should not be viewed as obstacles to research, but rather as the means through which human subject research is facilitated. There has been some proliferation of regulations applicable to human subject research, as well as of oversight mechanisms to ensure compliance. In addition, differing regulatory language governing FDA-regulated research versus HHS-sponsored research creates delays and confusion during the clinical research process.

- **Harmonize and standardize Federal policies pertaining to clinical research which promote the integrity and effectiveness of Federal and institutional systems of oversight. (FDA, NIH, HHS)**

The system for regulating human subject protections is embodied in the current Federal Policy for the Protection of Human Subjects in Research and in FDA regulations that apply to human subject research conducted to develop products currently regulated by the FDA. HHS, NIH, and the FDA should work with academic and industry researchers, as well as the patient advocacy community, to harmonize these regulatory requirements.

- **Develop a simplified informed consent template. (FDA, HHS)**

One of the most efficient mechanisms for speeding innovation will be to identify and expand the use of existing "best practices" that already have been developed by individual agencies. Starting with the work the NCI has done already to develop an informed consent template for use in cancer trials, the NIH, NCI, FDA, and OHPR should develop a simplified, uniform informed consent template. Such a template could help facilitate both patient and physician recruitment by simplifying enrollment procedures and IRB review by providing uniformity.

- **Streamline IRB process for large multi-center clinical trials. (HHS, NCI, NIH, FDA)**

HHS should create a mechanism for centralized IRB review in instances where there are multiple sites participating in a particular trial. The NCI and OHRP have created a pilot central IRB project that has been very successful. HHS in cooperation with the NCI, NIH, and the FDA should expand this pilot to cover non-cancer clinical trials and non-Phase III trials.

- **Clarify the applicability of the Health Insurance Portability and Accountability Act (HIPAA) to medical research and harmonize HIPAA privacy and confidentiality provisions with other human subject research regulations. (NIH)**

Since it went into effect in April 2003, the HIPPA Privacy Rule has created general confusion within the research community about what is an allowable use of human-based information. One concern is that HIPAA raises new barriers to releasing personal health information into large databases. OCR, OHRP, and NIH should clarify the relationship of HIPAA requirements to

4. *WHAT FORUMS SHOULD HHS USE TO SURVEY CONSTITUENTS ABOUT OBSTACLES TO INNOVATION (E.G., PUBLIC MEETINGS, CONTRACT RESEARCH, FOCUS GROUPS)?*

Significant resources already have been devoted to analyzing the obstacles to translating basic biomedical research into treatments. HHS' challenge is to eliminate those obstacles through a coordinated effort that affords relevant stakeholders a real opportunity to contribute to the decision making process.

There are numerous constituencies that must be included in the efforts to improve the biomedical research process, many of whom are already represented in organizations that already work with HHS and its agencies. Patient advocacy organizations, for example, are a key stakeholder in the innovation discussion. They can provide invaluable insight and can be useful in trial design and review, recruitment, access, development of informed consent processes, monitoring, and dissemination of research results. Once an individual is enrolled in a trial, patient support groups can greatly improve compliance and retention.

However, there are some constituencies who continue to be underrepresented in this process, and whose involvement is critical to any innovation effort. Of particular note is the need for increased involvement of minority communities in clinical research. Innovation initiatives must ensure increased, participation by minority populations in clinical research for scientific, medical, and ethical reasons. HHS should make particular effort to solicit the participation and expertise of representatives of minority physician associations, the medical schools of historically black colleges, and other medical facilities and associations that serve predominantly minority populations.

5. *HOW CAN THE PORTABILITY OF INFORMATION BETWEEN HHS AGENCIES BE OPTIMIZED?*

Internal government initiatives and government-sponsored projects are responsible for most of the currently available shared data resources in the biomedical community. New government initiatives supporting broad data sharing (e.g., caBIG at the NCI) continue in this tradition. Disease focused private foundations are increasingly recognizing the need for data sharing among their grantees to maximize the return on their research investment. However, health sciences lag far behind many other sectors in the application of information technology to clinical research data management. In the move towards an interoperable electronic medical records system, it is critical that the resulting information infrastructure is able to capture, analyze and share information essential to the research community.

existing human subject protections and develop a HIPAA compliant format for consenting to the repeated use for research purposes of information stored in medical record databases. OHRP, the Office of Civil Rights, and the NIH should continue working to clarify and provide guidelines for HIPAA implementation and to make sure these are well publicized and understood by the research community.

Facilitate the Development of "Personalized Medicine" Through Developing Appropriate Regulatory Approaches

Rapid advances in biology and information technology coupled with progress in the sequencing of the human genome offer new building blocks to advance medical science. The emerging field of personalized medicine has the potential to offer therapeutics tailored to an individual's unique genetic make-up. It also is anticipated that improved diagnostic and screening methodologies will facilitate recognition of an individual's predisposition towards a particular disease, thus facilitating early intervention and adoption of preventative measures. These new developments will require new regulatory approaches for assessing safety and efficacy. As this field develops, the FDA should work with the NIH, industry, and academic partners to develop effective guidance and decision criteria for reviewing these new therapies and diagnostic tools.

3. HOW CAN THE HHS SCIENTIFIC AND REGULATORY AGENCIES WORK MORE EFFECTIVELY WITH CMS TO ELIMINATE OBSTACLES TO DEVELOPMENT?

Under the current system, there are some inconsistencies in CMS's reimbursement process for drugs, therapies, and devices. While CMS will generally not reimburse for non-FDA approved medical therapies, there is no guarantee of CMS reimbursement for FDA-approved products. This creates a significant degree of uncertainty within the pharmaceutical, biotechnology, and medical device communities and may serve to limit innovation of new medical technologies.

Explore ways to modify the current federal reimbursement structure so as to encourage innovation

Recent reports, including the FDA's Critical Path Report, have noted a decrease in medical technology innovation. CMS in consultation with the FDA and perhaps AHRQ should explore using the powerful financial impact of its formulary listing to reward development of innovative medical therapies. For example, Europe and other countries have systems that reimburse use of "first-in-class" drugs with premium pricing. "Follow-on" drugs in a given class must demonstrate advantage over the first or they are not reimbursed. The implementation of this type of system might provide a needed boost to the innovation process within the United States and should be explored.

- **Require greater data-sharing efforts among and within the agencies themselves as well as between the agencies and the broader research community. (All HHS Agencies)**

All HHS medical record databases should be inventoried and made widely available, to the extent such databases contain information relevant for research and are not otherwise exempt from disclosure because of privacy or proprietary concerns. HHS also should inventory their own data archives and determine what steps are needed to promote greater interagency access to useful information (such as converting paper documents to electronic formats or standardizing electronic documents for optical character recognition). HHS should require the broadest access to data developed with federal support that is consistent with high quality research practice.

- **Support programs to increase data sharing within the industry, government, and academic communities. (NIH, FDA)**

The recent NIH Data Sharing Program is an excellent step towards creating data sharing incentives for the academic research community. Additionally, the FDA's requirement for industry to post on-line clinical trial information for drugs used to treat serious or life-threatening diseases and conditions will be an important step promoting greater clinical data sharing by the industry research community, once the requirement is appropriately implemented and enforced.

- **Fully integrate research needs into the mandate of the developing NHII Initiative. (HHS)**

Research needs must be fully integrated into the development of the NHII for that initiative to achieve its full potential. *FasterCures* encourages all parties to ensure that the important dual uses of electronic personal medical record systems for both healthcare and health research be fully realized as the NHII effort moves forward.

6. *WHICH HHS POLICIES AND PROGRAMS EFFECTIVELY SPUR INNOVATION? WHICH POLICIES AND PROGRAMS AT NIH (AND ITS GRANTEES), CMS, FDA, AND CDC SHOULD BE EXPANDED TO HELP SPUR INNOVATION? DO ANY POLICIES AND PROGRAMS POSE OBSTACLES TO INNOVATION?*

In recent years several HHS agencies have developed novel approaches to spur innovation and speed the introduction of new diagnostics and treatments into routine medical care. These programs have established best practices, streamlined reporting and oversight requirements, provided opportunities for collaboration across disciplines and institutions, and facilitated greater public input into setting research priorities.

The following HHS programs either have already spurred innovation or have potential to do so:

- AHRQ's TRIP Program.
- CDC's Futures Initiative.
- ClinicalTrials.gov if expanded into a comprehensive database, and monitored.
- FDA's Critical Path Initiative.

- FDA's Good Clinical Practice in FDA-Regulated Clinical Trials.
- FDA's Orphan Drug Program.
- FDA's 2002 requirement of on-line posting of clinical trial information if enforced and compliance measured.
- HHS' NHII Initiative.
- NIH/FDA Genetic Modification Clinical Research Information System (GeMCRIS).
- NIH's Roadmap Initiative.
- NIH's Data Sharing Program.
- NIH's Glue Grant Collaborative Research Program.
- NIH's educational-loan-relief program which provided $30 million to eligible clinical researchers.
- NIH's General Clinical Research Centers.
- NCI's caBIG Initiative.
- NCI's Clinical Trials Working Group.
- NCI's Clinical Trials Education Series.
- The pilot Central IRB project between the NCI and OHRP.

7. *WHAT ROLE SHOULD BE PLAYED BY NONGOVERNMENTAL PARTNERS IN ASSISTING THE FEDERAL GOVERNMENT IN THIS PROCESS?*

The academic, industry, patient, nonprofit advocacy and funding communities each play a critical role in the biomedical research endeavor. As such, they should participate in the development and implementation of HHS' innovation initiatives. These communities have considerable expertise to bring to bear in solving the many complex challenges that impede the translation of biomedical research into cures. Non-governmental partners should provide feedback, expertise, environments for piloting new initiatives, and appropriate resources (both human and financial capital) to assist the federal government in its efforts to stimulate innovation in medical technologies. HHS should explore creating public-private partnerships to harness the unique resources of non-governmental stakeholders in the innovation effort.

Below are some specific initiatives where non-governmental partners could support the government's efforts:

- **Partner with academic training institutions to ensure the availability of clinical researchers by creating new incentives to promote interest in clinical research.**

- **Initiate public education programs about clinical trial participation and programs to increase clinical trial enrollment in partnership with voluntary health associations, disease foundations, and public charities.**

- **Develop innovative research collaborations in partnership with disease foundations to conduct disease-specific research: develop novel disease models, undertake high risk research projects, and explore the therapeutic value of unpatentable compounds.**